Gaucher Disease Agents
(Elelyso, Cerdelga, Cerezyme, VPRIV, and Zavesca)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Gaucher Disease Agents policy is to ensure appropriate selection of patients for therapy based on product labeling, clinical guidelines and clinical studies while steering utilization to the most cost-effective medication within the therapeutic class. For this program, Cerezyme is the preferred product. Coverage for non-preferred products (Elelyso and VPRIV) is provided based on clinical circumstances that would exclude the use of the preferred product and may be based on previous use of a product. The coverage review process will ascertain situations where a clinical exception can be made. This program applies to all members requesting treatment with a non-preferred product for an indication that is also FDA-approved for the preferred product.

*Cerdelga and Zavesca are excluded from the preferred product requirement.

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
1. Elelyso is indicated for the treatment of patients with a confirmed diagnosis of type 1 Gaucher disease.
2. VPRIV is indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.
3. Cerezyme is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly.

Compendial Uses
Gaucher disease type 3 (Cerezyme only)

All other indications are considered experimental/investigational and are not a covered benefit.
Table. Gaucher Disease Agents

<table>
<thead>
<tr>
<th>Medication</th>
<th>Generic Name</th>
</tr>
</thead>
<tbody>
<tr>
<td>Preferred Products:</td>
<td></td>
</tr>
<tr>
<td>Cerezyme</td>
<td>imiglucerase</td>
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<tr>
<td>Targeted Products:</td>
<td></td>
</tr>
<tr>
<td>Elelyso</td>
<td>taliglucerase alfa</td>
</tr>
<tr>
<td>VPRIV</td>
<td>velaglucerase alfa</td>
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</tbody>
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POLICY

CRITERIA FOR INITIAL APPROVAL

I. Elelyso is considered medically necessary for treatment of Gaucher disease type 1 when the following criteria are met:
   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. Member is currently receiving treatment with Elelyso (excluding when obtained as samples or via manufacturer’s patient assistance programs) OR member has had a documented inadequate response or an intolerable adverse event with the preferred product Cerezyme.

Approval is for lifetime

II. Coverage for VPRIV is considered medically necessary for treatment of Gaucher disease type 1 when the following criteria are met:
   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. Member is currently receiving treatment with Elelyso (excluding when obtained as samples or via manufacturer’s patient assistance programs) OR member has had a documented inadequate response or an intolerable adverse event with the preferred product Cerezyme.

Approval is for lifetime

III. Coverage for Cerezyme is considered medically necessary for treatment of Gaucher disease type 1 and 3 when the following criteria is met:
   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

Approval is for lifetime

IV. Coverage for Cerdelga is considered medically necessary for treatment of Gaucher disease type 1 and 3 when the following criteria are met:
   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
   b. Member is a CYP2D6 extensive metabolizer, an intermediate metabolizer, or a poor metabolizer as detected by an FDA-cleared test

Approval is for 24 months
V. Coverage for Zavesca (miglustat) is considered **medically necessary** for treatment of Gaucher disease type 1 and 3 when the following criteria is met:

   a. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing

**Approval is for 24 months**

VI. Elelyso, VPRIV, and Cerezyme are considered not medically necessary for patients who do not meet the criteria set forth above.

CONTINUATION OF THERAPY

All members (including new members) requiring authorization for continuation of therapy must meet all initial authorization criteria.

**Prior approval is required.** Submit a prior approval/treatment request now.

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

PROCEDURES AND BILLING CODES

To report provider services, **use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.**

- J3060 – Inj, taliglucerace alfa 10 units (Elelyso)
- J1786 – Inj, imiglucerase, 10 units (Cerezyme)
- J3385 – Inj, velaglucerase alfa, 100 units (VPRIV)

REFERENCES
